HTA - - Health Technology Assessment Report

Impact of timing of PERT on gastrointestinal symptoms in Danish children and adolescents with CF.

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Study design (if review, criteria of inclusion for studies)

A systematic review of 79 clinical studies was conducted by independent researchers, funded by AHRQ. Studies of rhGH therapy in CF patients; reports of new discovery (specifically, randomized controlled trials, observational trials, systematic review/meta-analyses, or case reports).

Participants

7 key questions. Studies will be included in the evaluation of key questions 1, 2, 4, 6, and 7 if they are 1) studies of rhGH therapy, 2) conducted in CF patients, 3) studies report data on pre-specified clinical or humanistic outcomes 4) reports of new discovery (specifically, randomized controlled trials, observational trials, systematic review/meta-analyses, or case reports). Studies will be included in the key question 3 evaluation if they are 1) conducted in CF patients, 2) either randomized controlled trials or observational studies 3) report linkages between intermediate outcomes and health outcomes. Studies will be included in the key question 5 evaluation if they are 1) studies of rhGH therapy, 2) conducted in CF patients, idiopathic short stature, or growth hormone deficiency, 3) either randomized controlled trials or observational studies 4) studies report data on malignant outcomes.

Interventions

rhGH therapy

Outcome measures

Analytic framework mapping specific linkages from comparisons to subpopulations of interest, mechanisms of benefit, and outcomes of interest. It is a logic chain that supports the link from the intervention to the outcomes of interest. In patients with cystic fibrosis and relevant subgroups based upon gender, age, baseline clinical status, and prior therapy, we seek to answer the effect that intervention with rhGH may have. The first step in the analytic framework deals with intermediate outcomes from rhGH treatment, which includes IGF factors, protein turnover markers, nutritional status, growth measures, bone measures, lung function, pulmonary exacerbations, exercise tolerance, antibiotic use, sex hormones and pubertal development. Final health outcomes can either be answered from the direct evidence that exists in cystic fibrosis patients treated with rhGH or by assessing the link between intermediate and final health outcomes (which include health-related quality-of-life, hospitalization, bone fracture, or mortality). Adverse events associated with rhGH use are also evaluated, including altered glucose metabolism, development of diabetes mellitus, lymphoid overgrowth, or malignancy

Main results

Evidence Associating Intermediate Outcomes With Mortality Risk and HRQoL for Patients With CF: Only one measure of pulmonary function, %FEV1, is strongly associated with either mortality risk or some domains of HRQoL. Weight is associated with mortality risk, but its effect on HRQoL is not clear. Evidence About Benefits: The data from current studies are insufficient to permit conclusions about mortality and HRQoL for patients with CF who were treated with rhGH. Patients treated with rhGH experienced 1.6 fewer hospitalizations per year. Effect on intermediate outcomes: % FEV1 was not significantly improved by rhGH treatment. Weight, weight velocity, and BMI improved. Percent ideal body weight improved. Evidence About Harms: After the 6â€“12 month course of treatment typical in these studies, blood glucose was elevated (5.7 mg/dL over controls on average), but evidence is insufficient to evaluate the long-term risks of either CFRD or cancer. Treatment with rhGH appeared to be well tolerated as study withdrawals were rare

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See also

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Keywords

Growth Hormone; Hormones; pharmacological_intervention; Recombinant Proteins; rhGH;